

Title (en)

AAV-MEDIATED GENE THERAPY FOR RPGR X-LINKED RETINAL DEGENERATION

Title (de)

AAV-VERMITTELTE GENTHERAPIE ZUR X-CHROMOSOMALEN NETZHAUTDEGENERATION

Title (fr)

THÉRAPIE GÉNIQUE INDUITE PAR VAA S'APPLIQUANT À LA DÉGÉNÉRESCENCE RÉTINIENNE LIÉE À L'X DE RPGR

Publication

**EP 2872183 A4 20151209 (EN)**

Application

**EP 13816245 A 20130123**

Priority

- US 201261670355 P 20120711
- US 2013022628 W 20130123

Abstract (en)

[origin: WO2014011210A1] Described herein are methods of preventing, arresting progression of or ameliorating vision loss and other conditions associated with retinitis pigmentosa and x- linked retinitis pigmentosa in a subject. The methods include administering to said subject an effective concentration of a composition comprising a recombinant adeno -associated virus (AAV) carrying a nucleic acid sequence encoding a normal retinitis pigmentosa GTPase regulator (RPGR gene), or fragment thereof, under the control of regulatory sequences which express the product of the gene in the photoreceptor cells of the subject, and a pharmaceutically acceptable carrier.

IPC 8 full level

**A61K 48/00** (2006.01); **A61K 38/17** (2006.01); **A61K 38/46** (2006.01); **A61P 27/02** (2006.01); **C07K 14/47** (2006.01)

CPC (source: EP US)

**A61K 38/1709** (2013.01 - EP US); **A61K 38/185** (2013.01 - EP US); **A61K 38/46** (2013.01 - EP US); **A61K 48/005** (2013.01 - EP US); **A61P 27/02** (2018.01 - EP); **A61P 43/00** (2018.01 - EP); **C07K 14/4702** (2013.01 - EP US); **C12N 15/86** (2013.01 - US); **A61K 48/00** (2013.01 - US); **C12N 2750/14143** (2013.01 - EP US); **C12N 2830/008** (2013.01 - EP US); **C12Y 306/05** (2013.01 - EP US)

Citation (search report)

- [Y] US 2009202505 A1 20090813 - BARTUS RAYMOND T [US], et al
- [XY] M. NATKUNARAJAH ET AL: "AAV Mediated Gene Replacement Therapy in the RPGR Knockout Mouse- A Model of X-Linked Retinitis Pigmentosa", INVESTIGATIVE OPHTHALMOLOGY & VISUAL SCIENCE - IOVS, vol. 46, no. Suppl. S, 1 May 2005 (2005-05-01), US, pages 5224, XP055223624, ISSN: 0146-0404
- [Y] DONG-HYUN HONG ET AL: "A Single, Abbreviated RPGR-ORF15 Variant Reconstitutes RPGR Function In Vivo", INVESTIGATIVE OPHTHALMOLOGY & VISUAL SCIENCE, vol. 46, no. 2, 1 February 2005 (2005-02-01), US, pages 435, XP055218709, ISSN: 1552-5783, DOI: 10.1167/iovs.04-1065
- [Y] PANG ET AL: "Comparative analysis of in vivo and in vitro AAV vector transduction in the neonatal mouse retina: Effects of serotype and site of administration", VISION RESEARCH, PERGAMON PRESS, OXFORD, GB, vol. 48, no. 3, 22 October 2007 (2007-10-22), pages 377 - 385, XP022452232, ISSN: 0042-6989, DOI: 10.1016/J.VISRES.2007.08.009
- See also references of WO 2014011210A1

Designated contracting state (EPC)

AL AT BE BG CH CY CZ DE DK EE ES FI FR GB GR HR HU IE IS IT LI LT LU LV MC MK MT NL NO PL PT RO RS SE SI SK SM TR

DOCDB simple family (publication)

**WO 2014011210 A1 20140116**; AU 2013287281 A1 20150219; AU 2013287281 B2 20180426; CA 2878171 A1 20140116; CA 2878171 C 20210427; CN 105120901 A 20151202; EP 2872183 A1 20150520; EP 2872183 A4 20151209; EP 2872183 B1 20180926; JP 2015523379 A 20150813; JP 6199965 B2 20170920; NZ 704275 A 20160930; US 10383922 B2 20190820; US 2015202269 A1 20150723; US 2018036385 A1 20180208; US 9770491 B2 20170926

DOCDB simple family (application)

**US 2013022628 W 20130123**; AU 2013287281 A 20130123; CA 2878171 A 20130123; CN 201380045636 A 20130123; EP 13816245 A 20130123; JP 2015521593 A 20130123; NZ 70427513 A 20130123; US 201314413884 A 20130123; US 201715699262 A 20170908